

61 1. (thrice amended) A recombinant adeno-associated virus vector, which comprises:

- a) at least a portion of the adeno-associated virus genome; and
- b) at least one eukaryotic based nucleic acid sequence that encodes a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence chosen from the region located from about hypersensitive site I to about hypersensitive site VI of the human globin gene cluster, which is heterologous to the wild-type gene product, said virus vector having the property of regulating immune cell specific expression of said nucleic acid sequence or nucleic acid sequences upon stable transduction of a target mammalian immune cell.

62 2. (twice amended) A recombinant adeno-associated virus vector of Claim [7] 1 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located within the group of *cis*-acting regulatory sequences consisting of hypersensitive site I, hypersensitive site II, hypersensitive site III, hypersensitive site IV, and hypersensitive site VI, in association with the human globin gene.

63 3. (twice amended) A recombinant adeno-associated virus vector of Claim [4] 1 wherein said *cis*-acting regulatory sequence comprises hypersensitive site II, associated with the human globin gene cluster.

64 4. (twice amended) A recombinant adeno-associated virus vector of Claim [4] 1 wherein said immune cell is chosen from the group consisting of a human hemapoietic stem cell, a human myeloid progenitor cell and a human erythroid progenitor cell.

65 5. (twice amended) A recombinant adeno-associated virus vector of Claim [9] 1 wherein said [target] immune cell is K562.

66 ~~63~~ (twice amended) A recombinant adeno-associated virus vector of Claim [27] 1 which comprises a [DNA] nucleic acid sequence encoding a wild-type Fanconi anemia C complementing protein.

67 ~~73~~ (twice amended) A recombinant adeno-associated virus vector of Claim [27] 1 which comprises a [DNA] nucleic acid sequence encoding a wild-type Factor IX protein.

#### REMARKS

Claims 1, 4, 7-12, 16-17, 19-21, 25-32, 33, 39, and 46-48 were pending in the instant application. Applicants have canceled Claims 4, 7, 9-12, 16, 17, 21, 25-32, 47, and 48 without prejudice to the Applicants right to pursue the subject matter of the canceled claims in related applications. Claims 1, 8, 16, 19, 20, 33, and 39 have been amended to more particularly point out and distinctly claim the subject matter of the present invention. With entry of this amendment, therefore, Claims 1, 8, 16, 19, 20, 33, 39, and 46 will be pending. For the Examiner's convenience, a copy of the pending claims as amended is annexed hereto as Exhibit A.

#### **1. THE REJECTIONS UNDER 35 U.S.C. § 112 SHOULD BE WITHDRAWN**

Claims 27, 31, 33, 39, and 47 are rejected under 35 U.S.C. § 112, first paragraph, as containing subject matter which was not described in the specification in such a way as to reasonably convey to one skilled in the art that the Applicants, at the time the application was filed, had possession of the claimed invention. The Examiner contends that the specification provides no guidance as to regulatory elements specific for human primary hematopoietic cells other than the hypersensitive sites I-IV associated with the human globin gene cluster. Applicants have canceled Claims 27, 31, and 47 and have amended Claims 33 and 39 to be dependent from Claim 1. Claim 1 recites a recombinant AAV vector comprising a eukaryotic based nucleic acid sequence encoding a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence chosen from the region located from about hypersensitive site I to about hypersensitive site VI of the human globin gene cluster, which